

Supplementary table 2: miHTT expression in off-target organs a) NHP and b) rat

a)

Dose, gc/brain	miHTT expression in off-target organs in NHP (gc/μg of RNA)						
	Adrenal gland	Cervical lymph node	Heart	Kidney	Liver	Spleen	Ovaries
2x10 ¹²	n=6 <LLOQ	n=6 <LLOQ	n=6 <LLOQ	n=5 4.04x10 ³ (9.98x10 ²)	n=6 <LLOQ	n=6 <LLOQ	Not analyzed
7x10 ¹²	n=2 4.73x10 ³ (2.48x10 ³)	n=6 <LLOQ	n=1 5.11x10 ³	n=1 3.34x10 ³	n=2 4.79x10 ³ (1.51x10 ³)	n=6 <LLOQ	Not analyzed
2x10 ¹³	n=3 6.12x10 ³ (3.54x10 ³)	n=6 <LLOQ	n=1 4.22x10 ³	n=6 <LLOQ	n=4 5.57x10 ³ (1.38x10 ³)	n=6 <LLOQ	n=3 2.35x10 ⁴ (2.22x10 ⁴)

Data are mean (standard deviation). N=6 for all tissues except ovaries (N=3). Where lower, n represents positively determined concentration of vector DNA. LLOQ was 25 copies/reaction. gc, genome copies; LLOQ, lower limit of quantification; miHTT, micro RNA targeting *HTT*; NHP, non-human primates.

b)

Time, months post dose	miHTT expression in off-target organs in rats (gc/μg of RNA)					
	Adrenal gland	Cervical lymph node	Liver	Lung	Spinal cord	Spleen
1	n=6 1.80x10 ⁴ (1.14x10 ⁴)	n=1 2.86x10 ³	n=10 <LLOQ	n=10 <LLOQ	n=10 <LLOQ	n=10 <LLOQ
6	n=7 2.63x10 ⁴ (3.30x10 ⁴)	n=10 <LLOQ	n=10 <LLOQ	n=2 3.98x10 ³ (6.15x10 ²)	n=5 4.81x10 ³ (1.43x10 ³)	n=2 5.54x10 ³ (3.93x10 ³)

Data are mean (standard deviation). N=10 for all tissues. Where lower, n represents positively determined concentration of vector DNA. LLOQ was 2.5x10³ copies/μg of RNA. gc, genome copies; LLOQ, lower limit of quantification; miHTT, micro RNA targeting *HTT*.